

## Understanding Alternative Methods and Metrics for Value Assessment

It is now widely recognized that traditional methods and metrics of value assessment – including the quality-adjusted life year (QALY) – have significant shortcomings. This has led to well-intentioned development of other measures and approaches that developers assert to be nondiscriminatory and more patient-centered. However, each approach comes with tradeoffs, need for improvement, and inherent methodological flaws. No patient is average, and no measure of value should assume so.

Value assessment may seem technical, but there is growing awareness of its relevance to public policy, as seen in recent final rules governing Section 504 of the Rehabilitation Act and Section 1557 of the Patient Protection and Affordable Care Act. These rules acknowledge their potential for discrimination. Yet, recent CMS guidance explicitly expressed interest in using alternative approaches as part of drug price negotiations. Therefore, it is critical stakeholders understand these innovative approaches.

There is a longstanding protection against use of discriminatory value assessment tools in statute. The Affordable Care Act barred Medicare's use of QALYs and similar measures that discount the value of a life because of an individual's disability. The aim of this language was not to spur an effort to find "loopholes" allowing the government to use a single approach. As discussed on the Senate floor, the spirit of the provision was to protect vulnerable patients and people with disabilities from policies that "set national practice standards or coverage restrictions" and ensure research used to make decisions is focused on clinical outcomes.<sup>1</sup>

### Every measure has tradeoffs between conditions advantaged and disadvantaged.

- **Quality-adjusted life year (QALY):** Less value to life-extending treatments among patients whose baseline health-related quality of life is low, particularly people living with disabilities. More value to treatments achieving maximum quality of life.
- **Equal value of life year gained (evLYG):** Less value to treatments improving quality of life in extended life years. Same value as QALYs for treatments that do not extend life years regardless of quality-of-life improvements. More value to treatments extending life years.
- **Generalized Cost Effectiveness Analysis (GCEA):** Less value to treatments for common conditions to manage symptoms. More value to treatments for severe and disabling conditions.
- **Generalized risk-adjusted cost effectiveness (GRACE):** Less value to treatments for common conditions to manage symptoms. More value to treatments for severe and disabling conditions.
- **Disability adjusted life year (DALY):** Less value to treatments for people with disabilities due to focus on life years lost. More value to conditions leading to an early death without treatment.
- **Health years in total (HYT):** Less value to treatments that improve quality of life without increasing life expectancy. More value to treatments that extend life.
- **Life years gained (LYG):** Less value to treatments for patients with fewer years left to live (e.g., older adults or those with disabling conditions) and for largely non-fatal conditions (e.g., blindness, depression, rheumatoid arthritis). More value to treatments extending life.

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<sup>1</sup> Colloquy by Senators Baucus, Enzi, Conrad, Hatch, Carper and Menendez. "Comparative Effectiveness Research Funds." Congressional Record 155:24 (February 6, 2009) p. S. 1796.

**Value assessment is only as good as the data driving it.**

**Health utilities:** Also known as Health State Utility Value (HSUV), they mark the health-related quality of life (HrQOL) of a patient with a specific disease. A numeric valuation is applied to a health state based on preference of being in that state relative to perfect health, assigning a number between 0 and 1 to various conditions a person's health could be in (often called "health states" in which 0=death and 1=optimal health). They are typically derived from surveys asking how much, on average, someone prefers one health state compared to another. Health states typically represent degree of impairment (not the disability or condition) such as active disease, response, remission, or mild, moderate and severe. Shortcomings include:

- Survey data relies on average perspectives of quality of life in a health state, which are biased, inaccurate and almost never replicable. For example, there is significant research on the bias against disability among the public and among providers.
- The identified health states are typically not disease or condition specific, often surveying health from lens of mild, moderate or severe (such as the EQ-5D) and only accounting for health improvements that move between these broad states. Only large health improvements, i.e. HrQOL, count.
- Health utilities typically give a lower value to people living below optimal health. For example, extending the life for person living at a .5 is worth half of a person at a 1.

**Disability weights:** Disability weights quantify health losses relating to non-fatal outcomes, expressed as years lived with disability (YLD). They typically have a value between 0 (equivalent to full health) and 1 (equivalent to death). For example, living 10 years with a 10% reduction in HRQoL is a disability weight of 0.10 – equal to losing one full year of good health (e.g., by dying one year before the life expectancy). Severity of condition (morbidity) and its death rate (mortality) are expressed as the number of healthy life years lost. Shortcomings include:

- Disability weights are elicited by surveys, often of participants that do not have experience in the studied health state. Surveys are subject to bias against disability.
- Disability weights from different studies are often not comparable, coming from different countries or populations with differing perceptions of disease and disability.
- Assuming same weights to different aspects of quality of life as representative of all people risks being applicable to none. Triathletes may highly weigh physical function. Academics may weigh mental acuity.

**Health outcomes data:** Cost effectiveness analysis requires data on health outcomes to measure cost of gaining health. A product's "value" combines clinical effectiveness (impact of intervention on select health outcomes) and economic value (impact of intervention on healthcare resource use and costs). Yet, health data is lacking:

- Patient-centered outcomes and societal value are often ignored. For example, methods may not incorporate data on economic or social consequences such as loss of ability to work or caregiver effects.
- Reliance on average estimates based on generic survey data obscures important differences in clinical needs and preferences, particularly complex diseases and those from underrepresented communities.

**Health equity:** Cost effectiveness analyses and value assessment are intended to maximize health care efficiency. Historically, they have not explicitly incorporated equity concerns related to race, ethnicity, or socioeconomic factors, nor implicit bias or structural inequities within healthcare systems, disparities in access to healthcare services and treatments, or social determinants of health.

**Real-World Implications:** New methodologies for cost effectiveness analysis are abundant but untested. While recognition of flaws inherent in historic methods for assessing treatment value is driving innovation, literature on almost every method underscores need for extensive detailed data on patients' risk profiles, co-existing conditions, and other relevant factors currently lacking and challenging to obtain. Investment in data is needed.